INAHTA Brief

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Title KALYDECO – A Health Technology Assessment

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Reference link to full report in French
https://www.has-sante.fr/jcms/pprd_2984153/fr/kalydeco

Aim

Assessment of KALYDECO (ivacaftor) with a view to funding by the French national health insurance system and of its clinical contribution compared to other strategies in the indication treatment of infants aged at least 6 months, toddlers and children weighing 5 kg to less than 25 kg with cystic fibrosis (CF) who have one of the gating defect (class III) mutations in the CFTR gene mentioned in the MA.

Conclusions of the Transparency Committee

Clinical Benefit

- Cystic fibrosis is a serious disease that is life-limiting for patients.
- KALYDECO (ivacaftor) products are curative treatments.
- The efficacy/adverse effects ratio of KALYDECO (ivacaftor) in infants aged from more than 6 months to less than 1 year and weighing 5 kg to less than 25 kg is high.
- To date, there are no other treatments targeting the causes of the disease.
- KALYDECO (ivacaftor) products are 1st line treatments.
- KALYDECO (ivacaftor) is likely to have an additional impact on public health in infants aged at least 6 months to 1 year.

Considering all of the above, the clinical benefit of KALYDECO (ivacaftor) is substantial in the MA indication for the 25 mg strength and in the MA indication extension for the 50 mg and 75 mg strengths.

Clinical Added Value
Considering:
- an efficacy and safety of ivacaftor (KALYDECO) in children aged at least 6 months to 1 year similar to those already assessed by the Committee in children aged from 1 year and over to less than 2 years, based on:
- the safety profile of ivacaftor, which appears to be acceptable in children aged from at least 6 months to 1 year,
- the identified medical need in the absence of any other treatment targeting the causes of the disease,

and despite:
- the follow-up limited to 24 weeks of treatment not enabling evaluation of the long-term efficacy and safety,

KALYDECO (ivacaftor) provides important Clinical Added Value (CAV II) in the treatment of cystic fibrosis in infants aged at least 6 months to 1 year and weighing 5 kg to less than 25 kg who have one of the following gating defect (class III) mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N or S549R.

Recommendations

The Transparency Committee issued its approval for the funding of KALYDECO (ivacaftor) by the French national health insurance system (private practice and hospital) in the indication treatment of infants aged at least 6 months, toddlers and children weighing 5 kg to less than 25 kg with cystic fibrosis (CF) who have one of the gating defect (class III) mutations in the CFTR gene mentioned in the MA.

Methods

The assessment of KALYDECO (ivacaftor) was founded on evidence-based medicine with a critical analysis of the clinical data.

Written by

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